

|                               |                              |                     |  |
|-------------------------------|------------------------------|---------------------|--|
| <b>Notice of Allowability</b> | <b>Application No.</b>       | <b>Applicant(s)</b> |  |
|                               | 10/044,869                   | SHAYMAN, JAMES A.   |  |
|                               | Examiner<br>Janet L. Coppins | Art Unit<br>1626    |  |

-- The MAILING DATE of this communication appears on the cover sheet with the correspondence address--

All claims being allowable, PROSECUTION ON THE MERITS IS (OR REMAINS) CLOSED in this application. If not included herewith (or previously mailed), a Notice of Allowance (PTOL-85) or other appropriate communication will be mailed in due course. **THIS NOTICE OF ALLOWABILITY IS NOT A GRANT OF PATENT RIGHTS.** This application is subject to withdrawal from issue at the initiative of the Office or upon petition by the applicant. See 37 CFR 1.313 and MPEP 1308.

1.  This communication is responsive to Applicants' Amendment of April 28, 2005.
2.  The allowed claim(s) is/are 1, 2, 4, 7, 12, 13, 16, 17, 19, 24, 25, 27, and 36, newly renumbered as 1-13.
3.  The drawings filed on 10 January 2002 are accepted by the Examiner.
4.  Acknowledgment is made of a claim for foreign priority under 35 U.S.C. § 119(a)-(d) or (f).
  - a)  All
  - b)  Some\*
  - c)  None
 of the:
  1.  Certified copies of the priority documents have been received.
  2.  Certified copies of the priority documents have been received in Application No. \_\_\_\_\_.
  3.  Copies of the certified copies of the priority documents have been received in this national stage application from the International Bureau (PCT Rule 17.2(a)).

\* Certified copies not received: \_\_\_\_\_.

Applicant has THREE MONTHS FROM THE "MAILING DATE" of this communication to file a reply complying with the requirements noted below. Failure to timely comply will result in ABANDONMENT of this application.  
**THIS THREE-MONTH PERIOD IS NOT EXTENDABLE.**

5.  A SUBSTITUTE OATH OR DECLARATION must be submitted. Note the attached EXAMINER'S AMENDMENT or NOTICE OF INFORMAL PATENT APPLICATION (PTO-152) which gives reason(s) why the oath or declaration is deficient.
6.  CORRECTED DRAWINGS (as "replacement sheets") must be submitted.
  - (a)  including changes required by the Notice of Draftsperson's Patent Drawing Review (PTO-948) attached
    - 1)  hereto or 2)  to Paper No./Mail Date \_\_\_\_\_.
  - (b)  including changes required by the attached Examiner's Amendment / Comment or in the Office action of Paper No./Mail Date \_\_\_\_\_.

**Identifying indicia such as the application number (see 37 CFR 1.84(c)) should be written on the drawings in the front (not the back) of each sheet. Replacement sheet(s) should be labeled as such in the header according to 37 CFR 1.121(d).**

7.  DEPOSIT OF and/or INFORMATION about the deposit of BIOLOGICAL MATERIAL must be submitted. Note the attached Examiner's comment regarding REQUIREMENT FOR THE DEPOSIT OF BIOLOGICAL MATERIAL.

#### Attachment(s)

1.  Notice of References Cited (PTO-892)
2.  Notice of Draftsperson's Patent Drawing Review (PTO-948)
3.  Information Disclosure Statements (PTO-1449 or PTO/SB/08),  
Paper No./Mail Date \_\_\_\_\_
4.  Examiner's Comment Regarding Requirement for Deposit  
of Biological Material
5.  Notice of Informal Patent Application (PTO-152)
6.  Interview Summary (PTO-413),  
Paper No./Mail Date \_\_\_\_\_
7.  Examiner's Amendment/Comment
8.  Examiner's Statement of Reasons for Allowance
9.  Other \_\_\_\_\_

## **DETAILED ACTION**

Claims 1, 2, 4, 5, 7, 12, 13, 16, 17, 19, 24, 25, 27, and 36 pending in the instant application.

### *Response to Amendment*

1. Receipt is acknowledged of Applicants' Amendment, submitted April 28, 2005, which has been reviewed by the Examiner and entered of record in the file.
2. Accordingly, claims 3, 6, 8-10, 14, 15, 18, 20-22, 26, 28-30 and 32-35 have been cancelled, and claims 1, 7, 12, 19, 24, 27, and 36 have been amended.

### *Election/Restrictions*

3. Pursuant to the telephone conversations between Applicants' attorney, Steven G. Davis, and the Examiner, the Restriction Requirement has been overcome via Applicants' cancellations and amendatory changes to the claims.

### *Claim Rejections - 35 USC § 112*

4. Claims 3-5 and 35 previously rejected under 35 U.S.C. 112, second paragraph, as being indefinite. In view of Applicants' cancellations, the Examiner withdraws the rejections to the claims.

### *Allowable Subject Matter*

5. Claims 1, 2, 4, 5, 7, 12, 13, 16, 17, 19, 24, 25, 27, and 36, are allowable, as newly renumbered as claims 1-13.

The following is an examiner's statement of reasons for allowance: This invention relates to novel amino ceramide-like compounds that inhibit glucosylceramide (GlcCer) formation, and their methods of use. The allowable compounds in this case are limited to those according to the formulae of claims 1, 12, and 24, wherein R<sup>3</sup> is a cyclic tertiary amine (for example, piperidine,

Art Unit: 1626

morpholine, pyrrolidine, azetidine, and aziridine). The allowable methods are limited to methods of treating Gaucher's disease, Tay Sachs disease, Fabry's disease, Sandhoff disease, and GM1 gangliosidosis. Applicants demonstrate the effects of inhibiting GlcCer synthase activity in the numerous journal articles listed on pages 1-2 of the specification, as well as the effects of inhibiting GlcCer for treating patients suffering from genetic errors in the metabolism of GlcCer (for treating the above diseases, for example) on pages 9-12 of the specification. The claimed compounds are found allowable because certain ceramide-like compounds are known to those skilled in the art as useful agents for inhibiting GlcCer synthase activity, however the aspect of preparing the instant compounds, that contain a cyclic tertiary amine in the R<sup>3</sup> position and an aliphatic chain of 10 to 18 carbon atoms in the R<sup>2</sup> position, is novel and unobvious. The closest of prior art fails to teach or render obvious the preparation of the aforementioned compounds that contain the same ceramide-like structure as described above. For example, U.S. Patent No. 5,916,911 discloses similar ceramide-like compounds and their methods of use, however there is no overlap with the instantly claimed compounds since the '911 patent teaches a hydroxy group adjacent to the R<sup>1</sup> position, which differs from the instant claims since R<sup>4</sup> is an in vivo hydrolyzable group. Therefore, the prior art of record does not teach or fairly suggest the instant claimed compounds or their utility as agents for inhibiting the activity of GlcCer synthase.

Any comments considered necessary by applicant must be submitted no later than the payment of the issue fee and, to avoid processing delays, should preferably accompany the issue fee. Such submissions should be clearly labeled "Comments on Statement of Reasons for Allowance."

***Telephone Inquiry***

Any inquiry concerning this communication or earlier communications from the examiner should be directed to Janet L. Coppins whose telephone number is 571.272.0680. The examiner can normally be reached on M-F 8:30-5:00.

If attempts to reach the examiner by telephone are unsuccessful, the examiner's supervisor, Joseph K. McKane can be reached on 571.272.0699. The fax phone number for the organization where this application or proceeding is assigned is 571.272.8300.

Information regarding the status of an application may be obtained from the Patent Application Information Retrieval (PAIR) system. Status information for published applications may be obtained from either Private PAIR or Public PAIR. Status information for unpublished applications is available through Private PAIR only. For more information about the PAIR system, see <http://pair-direct.uspto.gov>. Should you have questions on access to the Private PAIR system, contact the Electronic Business Center (EBC) at 866-217-9197 (toll-free).

Janet L. Coppins  
July 7, 2005

**KAMAL A. SAEED, PH.D.  
PRIMARY EXAMINER**

 Kamal Saeed  
Joseph K. McKane  
Supervisory Patent Examiner, Art Unit 1626